CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

2008 Strategic Plan Update
Industry Section Stakeholder Draft – 12-1-08

SECTION 2. COLLABORATING WITH INDUSTRY

As the effort to cure or treat disease using stem cell technologies moves closer to the clinic, CIRM will need to call upon expertise in clinical trials, regulatory requirements, and large-scale Good Manufacturing Practices (GMP). The greatest reservoir of these skills resides in pharmaceutical and biotechnology companies. Without the injection of such expertise into CIRM programs, either independently or through formal collaborations between industry and universities and other not-for-profit organizations, CIRM's ability to accomplish its mission will be jeopardized. Therefore, one of CIRM's primary goals is to promote and facilitate the involvement of corporations in CIRM's programs so as to harness the resident expertise and resources in regulatory, clinical, manufacturing, and R&D arenas.

To achieve these goals, CIRM proposes four strategies:

Strategy 1: Ensure that CIRM's internal programs, policies and regulations embrace industry participation. In each aspect of its work, CIRM will be mindful of the benefits of industry participation as well as the impact of CIRM policies and practices on such participation, removing obstacles where appropriate. Towards that end, CIRM must ensure that:

- Its grant programs are conducive to industry participation, amending them as necessary to facilitate collaboration with pharmaceutical and biotech companies.
- CIRM's business loan program is implemented on terms that encourage industry participation while including provisions to assure preferential pricing for California local and state government entities and access to the uninsured.
- CIRM's rigorous peer review encourages industry participation. For example, by including on the Grants Working Group industry experts who are qualified to assess science and who recognize what is necessary for commercial success.

Strategy 2: Reach out to the pharmaceutical and biotechnology industry to better understand its needs and encourage its participation in CIRM programs. CIRM recognizes that the not-for-profit and business communities, while sharing the goal of using stem cell research to improve the lives of patients, have different missions, capabilities, and cultures. To promote industry involvement, CIRM must:

- Establish a "Biotech Advisory Group" comprised of representatives from the pharmaceutical and biotechnology sector to advise CIRM on industry trends and needs and to help evaluate CIRM's interactions with this sector.

- Join or establish a formal liaison with industry groups with a stake in stem cells at the state, national, and international levels, such as BIO, PhRMA, BayBio and BIOCOM**.**
- Include, as appropriate, topics with industry focus in CIRM-hosted and sponsored events and make sure industry representatives are included as participants and speakers.
- Take visible leadership roles at conferences, panels, and professional meetings addressing industry participation in stem cell research.

Strategy 3: Serve as a resource to support industry involvement in stem cell research and development. CIRM should provide:

- Tools and access to resources that are not easily obtainable from other sources and that are valuable to industry.
- Opportunities and guidance for joint collaborative projects involving industry and not-for-profit entities.
- Information and updates on best practices in intellectual property (IP) licensing and trade secret issues.
- Information on, and guidance in, grant writing and best practices in CIRM-specific grant management.
- Information on IP prosecution and other relevant legal developments. CIRM will consider establishing a network or panel of IP and licensing attorneys with strong client bases in the commercial stem cell arena. The group could meet periodically to share best practices and advise the Agency.
- Help in both monitoring "blockers" to industry participation and using CIRM resources and influence judiciously to resolve logjams. Examples might include reviewing the European Union's views on patentability of stem cell inventions and their impact on the commercial sector, and identifying factors that have led to failures in exploiting stem cell technology and recommending solutions.
- Opportunities to facilitate the exchange of information between companies regarding stem cell manufacturing and regulatory experience, with the aim of avoiding duplication of effort and repetition of mistakes. CIRM regulations already require the exchange of certain types of materials, once results are published by grantees; the Agency should monitor that exchange to ensure its benefits are being maximized.
- Advocacy for policy changes and clarifications of various state and federal agency rules that hinder industry involvement in stem cell research and development. Such advocacy might involve submitting *amicus* briefs in various legal proceedings, submitting "white papers" to government agencies, and testifying before governmental bodies.

Strategy 4: Educate key stakeholder constituencies about industry's critical role in accomplishing CIRM's mission. Key constituencies should be educated about the close links and cascade effects between health/economic benefits for Californians on the one hand, and industry innovation/intellectual property protection on the other. To improve understanding, CIRM should:

- Regularly update the Governing Board about relevant developments in the pharmaceutical and biotechnology sector.
- Help raise public awareness about biotechnology's unique ability and extensive track record of success in bringing valuable therapies to patients and the need for industry involvement to help drive similar stem-cell based therapeutics. This education can be implemented through information on CIRM's Website and in its annual report, publications and statements, and public events.
- Raise the awareness of state legislators about the importance of industry involvement in CIRM's mission.
- Encourage other funding organizations, especially those with whom we have established memoranda of understanding (MOUs), to fund industry research and development efforts.

Strategy 5: Providing loans to support later stages of research leading to clinical trials and new therapies. Bringing new and more effective treatments and therapies into practice is a complex, expensive process, and financing is becoming more difficult for small biotech companies. Since CIRM believes these companies will be important partners for California's world class academic and non-profit research institutions in bringing stem cell therapies to market, finding ways to support their efforts is essential.

CIRM is already providing opportunities for for-profit companies to apply for support of basic science research through its grant programs. In the next year the Agency will introduce a loan program that will provide significant new funding to biotech companies while maximizing CIRM's ability to achieve its goals by recycling monies re-paid from loans into new research programs. Key features of the loan program:

- RFAs for preclinical and clinical development will make loans available for for-profit companies.
- Applications for loans will undergo the same rigorous peer-review process as applications for grants.
- Standards will be developed for assessing financial feasibility and risks associated with loans.
- Underwriters will be identified to manage the financial aspects of the loan program.
- Loan funds repaid to CIRM will be recycled and used to support additional research RFAs.

To summarize, California's biotechnology and pharmaceutical companies are among the best in the world in their ability to develop innovative technologies and products to meet the needs of patients. In collaboration with Caliofrnia's leading non-profit research sector, they represent an invaluable and essential resource to bring stem cell therapies to patients; and they are enthused about CIRM and eager to contribute to the Agency's success. Working together, CIRM and its industry partners have unlimited potential to realize CIRM's lifesaving mission. To maximize this potential, CIRM must remain aware of the sector's specific needs for technology

and information and work diligently to provide opportunities for industry to engage – without hurdles -- with both CIRM and its not-for-profit partners.